Three UC's Join Forces to Launch CRISPR Clinical Trial Targeting Sickle Cell Disease

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Oakland, CA – The University of California, San Francisco (UCSF), in collaboration with UC Berkeley (UCB) and UC Los Angeles (UCLA), have been given permission by the US Food and Drug Administration (FDA) to launch a first-in-human clinical trial using CRISPR technology as a gene-editing technique to cure Sickle Cell Disease.

This research has been funded by CIRM from the early stages and, in a co-funding partnership with the National Heart, Lung, and Blood Institute under the Cure Sickle Cell initiative, CIRM supported the work that allowed this program to gain FDA permission to proceed into clinical trials.

Sickle Cell Disease is a blood disorder that affects around 100,000 people, mostly Black and Latinx people in the US. It is caused by a single genetic mutation that results in the production of "sickle" shaped red blood cells. Normal red blood cells are round and smooth and flow easily through blood vessels. But the sickle-shaped ones are rigid and brittle and clump together, clogging vessels and causing painful crisis episodes, recurrent hospitalization, multi-organ damage and mini-strokes.

The three UC's have combined their respective expertise to bring this program forward.

The CRISPR-Cas9 technology was developed by UC Berkeley's Nobel laureate Jennifer Doudna, PhD. UCLA is a collaborating site, with expertise in genetic analysis and cell manufacturing and UCSF Benioff Children's Hospital Oakland is the lead clinical center, leveraging its renowned expertise in cord blood and marrow transplantation and in gene therapy for sickle cell disease.

The approach involves retrieving blood stem cells from the patient and, using a technique involving electrical pulses, these cells are treated to correct the mutation using CRISPR technology. The corrected cells will then be transplanted back into the patient.

UCSF's Dr. Mark Walters, the principal investigator of the project, says using this new gene-editing approach could be a game-changer. "This therapy has the potential to transform sickle cell disease care by producing an accessible, curative treatment that is safer than the current therapy of stem cell transplant from a healthy bone marrow donor. If this is successfully applied in young patients, it has the potential to prevent irreversible complications of the disease. Based on our experience with bone marrow transplants, we predict that correcting 20% of the genes should be sufficient to out-compete the native sickle cells and have a strong clinical benefit."

Dr. Maria T. Millan, President & CEO of CIRM, said this collaborative approach can be a model for tackling other diseases. "When we entered into our partnership with the NHLBI we hoped that combining our resources and expertise could accelerate the development of cell and gene therapies for SCD. And now to see these three UC institutions collaborating on bringing this therapy to patients is truly exciting and highlights how working together we can achieve far more than just operating individually."

The 4-year study will include six adults and three adolescents with severe sickle cell disease. It is planned to begin this summer in Oakland and Los Angeles.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

About UCSF: The University of California, San Francisco (UCSF) is exclusively focused on the health sciences and is dedicated to promoting health worldwide through advanced biomedical research, graduate-level education in the life sciences and health professions, and excellence in patient care. It includes UCSF Health, which comprises three top-ranked hospitals, as well as affiliations throughout the Bay Area. Learn more at ucsf.edu, or see our Fact Sheet.

About UC Berkeley: UC Berkeley is the world's premier public university with a mission to excel in teaching, research and public service. This longstanding mission has led to the university's distinguished record of Nobel-level scholarship, innovation, a concern for the betterment of our world, and consistently high rankings of its schools and departments. One Nobel Prize-winning discovery, CRISPR-Cas9 gene editing, is revolutionizing life science research and disease treatment through the Innovative Genomics Institute, founded by the co-inventor of the technology.

About UCLA: One of the world's preeminent public research universities, UCLA is an international leader in breadth and quality of academic, research, health care, wellness, cultural, continuing education and athletic programs, with approximately 5,100 faculty members who teach nearly 46,000 students in the UCLA College and 12 professional schools. UCLA is consistently ranked among the top institutions nationally for research funding, having generated more than $1 billion in research grants and contracts annually in nine of the last 11 years.

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